Cautionary Statement

This presentation contains "forward-looking statements," as that term is defined under the Private Securities Litigation Reform Act of 1995 (PSLRA), which statements may be identified by words such as “expects,” “plans,” “projects,” “will,” “may,” “anticipates,” “believes,” “should,” “intends,” “estimates,” “potential” and other words of similar meaning, including statements regarding our estimated revenues and financial projections, our ability to achieve high levels of growth, the potential for our products under development to be game-changing, the potential of our next generation prostate markers to reduce biopsies and increase detection of cancer, our ability to develop a lead Alzheimer’s test and additional diagnostic products, the ability of our Alzheimer’s diagnostic test to distinguish Alzheimer’s disease from other dementias, our ability to develop certain therapeutics, new drugs and vaccines, including for the treatment of genetic diseases, cancers, neurological and metabolic disorders, our ability to develop and commercialize compounds that prevent the progression of neurodegenerative diseases, our ability to develop, test and launch new products, the expected timing of the studies and trials relating to our products under development, the outcome of our clinical trials and validation studies and that such outcomes will support commercialization, the expected size of the market for certain of our products under development, the potential benefits of our products under development, our ability to successfully develop and commercialize our product candidates such as simple blood tests for Alzheimer’s, Neuromyelitis Optica, Multiple Sclerosis and other neurological disorders, lung, pancreatic, and other cancers and autoimmune diseases, as well as products for other markets such as urology, women’s health, cardiology and infectious disease, our ability to develop and commercialize next generation prostate markers, and the timing of clinical trials and expected U.S. and European regulatory approvals for our product candidates and the commercial launch of our product candidates, as well as other non-historical statements. These forward-looking statements are only predictions and reflect our views as of the date they were made, and we undertake no obligation to update such statements. Such statements are subject to many risks and uncertainties that could cause our activities or actual results to differ materially from the activities and results anticipated in forward-looking statements, including risks inherent in funding, developing and obtaining regulatory approvals of new, commercially-viable and competitive products and treatments, general market factors, competitive product development, product availability, federal and state regulations and legislation, delays associated with development of novel technologies, unexpected difficulties and delays in validating and testing product candidates, the regulatory process for new products and indications, manufacturing issues that may arise, the cost of funding lengthy research programs, the need for and availability of additional capital, the possibility of infringing a third party’s patents or other intellectual property rights, the uncertainty of obtaining patents covering our products and processes and in successfully enforcing them against third parties, and the possibility of litigation, among other factors, including all of the risks identified under the heading Risk Factors in our Annual Report on Form 10-K and other filings with the Securities and Exchange Commission.
Company Overview

Opportunistic pharmaceutical and diagnostics company focused on large, high growth markets:

- New PLATFORM to develop diagnostic tests for cancer and neurodegenerative diseases
- New microfluidics SYSTEM for rapid, lab-quality point of care tests
- Next generation prostate cancer markers
- New neuroprotective drugs for neurodegenerative diseases.
- New drugs based on gene up-regulation, e.g. genetic diseases
- New drugs for asthma, COPD and cystic fibrosis
- Emerging markets pharmaceutical business
- Investments in other hi-tech companies

• Cash, cash equivalents and marketable securities of approximately $62 million as of 3/31/2012
Rolapitant Overview

- **Rolapitant out-licensed to Tesaro in December 2010**
  - Schering-Plough (S-P) divested to OPKO as FTC condition for S-P merger with Merck
  - Payments of up to $121MM
  - Double digit tiered royalties

- **Potentially best-in-class cancer supportive care product**
  - Potent neurokinin-1 (NK-1) receptor antagonist for chemotherapy-induced nausea and vomiting (CINV)
    - Single dose
    - Rapid onset
    - Long acting

- **Phase 3 program initiated following positive Phase 2 results**
  - Efficacy and dose identified in Phase 2 trial (n=454)
    - Five-day activity following single oral dose in patients treated with highly emetogenic chemotherapy (HEC)
  - Over 1,000 patients and healthy volunteers evaluated in Phase 1 and 2
  - Well accepted regulatory endpoints and clinical trial designs
    - Three global Phase 3 studies ongoing; 2 HEC (n=530) and 1 MEC (n=1350) with results expected during second half of 2013
Rolapitant Market Opportunity

- **U.S. market opportunity of ~ $1.25 billion**
  - 6.6 million annual CINV patient treatments in 2011
  - NCCN and ASCO guideline recommendations could lead to 70% penetration by the NK-1 class
  - Merck - EMEND® currently only NK-1 receptor antagonist on market
    EMEND® 2011 global sales - $419 million

- **Strong IP portfolio with U.S. exclusivity expected through 2028**
AntagoNAT
Natural Antisense Transcript Inhibitors

- Chemically modified single-strand oligonucleotides
- Up-regulate expression of endogenous proteins
- Extensive screening of over 400 gene targets
- Demonstrated up-regulation of genes related to genetic diseases, cancer, neurological, metabolic disorders
- Animal studies confirm increases of
  - Target mRNA
  - Proteins expressed by the target gene
- 8 AntagoNATs have been tested in animals
New Class of Compounds Prevent Neurodegenerative Disease Progression

• Licensed world-wide rights from The Scripps Research Institute to novel compounds that inhibit jun-N-terminal kinases (JNK) (important for neuron survival).

• Lead compound, a small molecule (SR-3306), could be first drug to protect brain in Parkinson’s disease, MS, ALS, and AD.

• Current therapies are symptomatic, have untoward side effects, lose effectiveness over time, or have risks associated with surgery.

• Inhibition of JNK to treat PD would be paradigm shifting by preventing neurodegeneration and disease progression.
Therapeutic Potential of JNK Inhibitors

- Orally bioavailable, brain penetrant JNK inhibitors have shown efficacy in two animal models of Parkinson’s disease and represent first in class neuroprotective agents for the treatment of PD.

- These JNK inhibitors have potential in all neurodegenerative disease including AD, MS, ALS, and stroke.
Antigen Surrogate Technology

B Cells

Foreign Antigen

Amplification

$10^5$-$10^6$ Synthetic Molecules

IgG antibody

(a) Antigen-binding site

$V_L$ domain

$V_H$ domain

$C_L$ domain

$C_H1$

$C_H2$

$C_H3$

Carbohydrate chain

Heavy chains

OPKO
Second Generation Screening System For Larger Libraries

Bead Library ($\approx$ one million compounds)

IgG ligands $\xrightarrow{\text{Healthy serum sample}}$

Denuded Library (still almost one million compounds)

Disease-specific IgG ligands

Cleave, spot to microarray $\xrightarrow{\text{Disease serum sample}}$

Denuded Library (still almost one million compounds)

Validate on many samples

Diagnostically Useful Peptoid-IgG Complexes
Neuromyelitis Optica (NMO)

- An autoimmune disorder of the CNS where autoantibodies destroy cells in the spinal cord and/or optic nerve.

- Often mis-diagnosed as multiple sclerosis, with serious adverse consequences.

- Autoantibodies recognize AQP4 on the surface of target cells in \(\approx 70\%\) of NMO patients. Recruits complement to destroy them.

- No simple blood test. Current diagnostics cumbersome and inefficient. Incapable of detecting \(\approx 30\%\) of anti-AQP4 antibody negative patients.
Discovery of Peptoid Surrogate of AQP4

1. Control Serum
2. NMO Serum

Spot Onto Slides In Triplicate

1. Probe With Monoclonal Antibody
2. Detect With Red-Labeled Anti-Human Ab

SH109  SH108
SH106  SH105

Anti-AQP4 Monoclonal Antibody

SH109  SH108
SH106  SH105

Control Monoclonal Antibody

(SH108 Binds Secondary Antibody)
Sensitive and Specific Detection of NMO

SH109  SH108
Normal Serum #6
No Anti-AQP4 Ab

SH106  SH105

SH109  SH108
NMO Serum #30
Anti-AQP4 Ab Positive

SH106  SH105

SH109  SH108
NMO Serum #74
Anti-AQP4 Ab Negative

SH106  SH105

10/10 Controls
10/10 NMO
Anti-AQP4+
8/10 NMO
Anti-Aqp4-
Development of A Commercially Viable ELISA-like Assay

![Chemical Structure]

- Linker
- Peptoid
- HRP
- Colorless substrate
- Blue Product

![Graph]

SH 106

- NMO
- NC
- MS
Plans - NMO

1. Identify higher affinity derivatives of SH106 and other peptoids. Akin to movement of a screening hit to a drug lead.

2. Complete a blinded study of ≈ 300 patients using the ELISA test to determine diagnostic sensitivity and specificity.

3. Optimize test on both Claros point of care platform and standard central laboratory ELISA platform.

4. Discovery has started on multiple sclerosis. NMO and MS diagnostic tests may be marketed as a package.
Lung Cancer Screening Protocol

1. Normal Non-smoker
   - Pool of 3 non-smoker serum samples
   - (~ 300 beads Removed)

2. Healthy Smoker
   - Pool of 5 Healthy Smoker serum samples
   - (~ 150 beads Removed)

3. Small Cell Lung Cancer
   - Pool of 3 Stage I SCLC serum samples
   - (70 beads Removed)
   - LCP6

4. Adenocarcinoma Lung Cancer
   - Pool of 3 Stage I Adenocarcinoma serum samples
   - (50 beads Removed)
   - LCP5

5. Squamous Cell Lung Cancer
   - Pool of 3 Stage I Squamous cell serum samples
   - (40 beads Removed)

Library Recycled for next disease
Microarray Validation of LCP6

(Disease Stage)

Serum Samples
Progress On Alzheimer’s Disease (AD)


Mid-2011: Completed validation study of 105 patients by microarray for ADPs 1-3.

Mid-2011: Began working on ELISA-like assay due to low throughput of microarray platform.

Mid-2011: Used second-generation bead screening technology to discover peptoids that bind new AD antibodies.

Dec. 2011: Initiated blinded microarray study of 120 samples, including MCI, in collaboration with BMS to determine efficacy of test for detection of very early AD. In progress.

Jan. 2012: Initiated efforts to improve affinity of peptoids to improve ELISA. In progress.

March 2012: Signed agreement with LabCorp to develop and market commercial AD test.

May 2012: Detected AD antibodies in cerebrospinal fluid of AD patients by ELISA.

June 2012: Will complete blinded ELISA-based analysis of 150 samples.
Blinded ELISA Study of 80 Samples

Clinical AD: 72% Agreement with OPKO test
Age-Matched Controls: 86% Agreement with OPKO test
Status Of Biomarker Discovery Projects

Samples Acquired/Acquiring: Ovarian Cancer, Prostate Cancer, Parkinson’s, TB, Malaria

Discovery (Library Screening): Dengue Fever, Multiple Sclerosis, Pancreatic Cancer, Colon Cancer, Narcolepsy

Initial Validation (Microarray): NMO, Lung Cancer

First-Generation ELISA Assay: NMO

Extensive Validation By ELISA: Alzheimer’s

Peptoid Optimization: Alzheimer’s

Extensive Validation of Optimized Peptoids by ELISA:
OPKO Diagnostics Platform

- **Product:** Immunoassay System Yielding Rapid Lab-Quality Quantitative Test Results with Finger-Stick Drop of Blood (OPKO Dx Card)

- **Technology**
  - Acquired Claros Diagnostics in October 2011
  - Proprietary Microfluidics and Amplification Chemistry
  - Pre-loaded Reagents on a Disposable Cassette
  - Sensitive Single Marker or Multiplex Assays
  - Inexpensive Analyzer

- **Initial Target Applications**
  - Urology / CE Mark Approval in Europe / US Clinical Trial Underway
  - Next Generation Prostate Cancer Markers (4Kscore™)

- **Specialty Tests:** Vitamin D

- **OPKO Molecular Diagnostic Tests Applications**

- **Strong IP Portfolio**
**OPKO Dx Card Technology Applications**

### Urology
- **PCa Panel:**
  - total-PSA
  - free-PSA
  - Testosterone
  - Novel Markers

### Women’s Health
- **Panel Tests:**
  - Pre-term bleed
  - Fertility
  - Menopause

### Cardiology
- **Cardio Panel:**
  - CTnI
  - BNP
  - D-dimer

### Infectious Disease
- **STD Panel:**
  - HIV
  - Hepatitis B
  - Syphilis

### GPs
- **Key Tests:**
  - TSH, T4
  - Vitamin D
  - Folic Acid

### Other

### Liquid Sample Capabilities

<table>
<thead>
<tr>
<th>Whole Blood</th>
<th>Semen</th>
<th>Spinal Fluid</th>
</tr>
</thead>
<tbody>
<tr>
<td>Serum</td>
<td>Saliva</td>
<td>Tears</td>
</tr>
<tr>
<td>Urine</td>
<td>Amniotic Fluid</td>
<td>Sweat</td>
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Overview of OPKO Diagnostics Technology

Key Attributes – High Quantitative Performance / Low Cost

- **Inexpensive Disposable**
  - Injection-Molded Microfluidics
  - On-Board Delivery of Multiple Reagents
  - Multiplex Platform

- **Robust Signal Detection**
  - Sensitive Proprietary Amplification Chemistry for Microfluidics

- **Inexpensive Hardware Unit (OPKO Dx Reader)**
  - Reliable Standard Optics

- **Ease of Use and Safety**
  - No Sample Prep
  - User-Friendly Sample Introduction
  - Biohazard Containment
Easy to Use

1. Collect Blood
2. Snap into Cassette
3. Insert into OPKO Dx Reader
4. Read Results in less than 10 minutes
OPKO Diagnostics
2012 Plans

- Urology
  - U.S. FDA Trial Completion / Filing
  - Proprietary Next Generation Prostate Markers
  - Signed license agreement in May, 2012 for launch of OPKO’s novel panel of Kallikrein markers in UK, Ireland, Sweden and Denmark
  - $\approx 70$ Million WW Tests Per Year $\approx$ $2$ Billion Market

- Vitamin D
  - EU Approval Expected in 2012
  - U.S. Approval Expected in 2013
  - Complementary Multiplex Panel Potential
  - $\approx 100$ Million WW Tests Per Year $\approx$ $5$ Billion Market
Next Generation Prostate Markers (4Kscore™)

• 1.2 Million prostate biopsies performed a year in U.S.
  – 750,000 of these are unnecessary
  – Costs in excess of $2.5 billion

• 4Kscore™ combines PSA and two novel kallikrein markers to provide significantly greater accuracy
  – Tested in over 10,000 patients to predict the probability of cancer-positive biopsy
  – Can dramatically reduce, up to 60%, of unnecessary biopsies now performed
OPKO Emerging Markets

**OPKO Chile** — Pharma Genexx, S.A.
- Rapidly growing sales from >100 products
- Revenues: $21.5 MM and $18.0 MM for 2011 and 2010, respectively
- Acquired ALS Distribution Limited, the exclusive product distributor of Arama Laboratories, in April, 2012
- Estimated 2012 Revenues of $26-$30 MM

**OPKO Mexico** — Pharmacos Exakta S.A. de C.V.
- 25 products across a range of therapeutic indications
  - Primarily branded ophthalmics, with expanding proprietary focus
- Revenues: $6.4 and $3.8 MM for year end 2011 and 2010, respectively

**OPKO Israel** — Fine Tech Pharmaceuticals, Ltd.
- Acquired in December, 2011
- Develops and produces high value, high potency active pharmaceutical ingredients
- FDA registered state of the art facility in Nesher, Israel
- Revenue $1.6 MM for the first three months ended March 31, 2012
Strategic Investments

Proprietary technologies with significant upside potential

CoCrystal Discovery, Inc. (~16% equity interest*)
- Founded by Nobel Laureate, Roger Kornberg, Ph.D.
- New approach to develop broad spectrum anti-viral drugs
- Development program with Teva for Hepatitis C drug

Sorrento Therapeutics (~23% equity interest*)
- New technology to produce human monoclonal antibodies libraries that are more complete and more efficient

Fabrus, LLC (~13% equity interest*)
- Next gen technology to identify therapeutic antibody targets
- Founded by Vaughn Smider, M.D., Ph.D. Scientist at The Scripps Research Institute

Tesaro, Inc. (~2% equity interest*)
- Oncology-focused biopharmaceutical company
- Founded by former executives of MGI Pharma
- Currently in registration for initial public offering

Neovasc, Inc. (~4% equity interest*)
- Developing innovative vascular devices
  - Reducer™ for refractory angina undergoing multicenter clinical trial
  - Tiara™ pre-clinical novel transcatheter mitral valve

ChromaDex, Inc. (~2% equity interest)
- Dietary supplement BlueScience and antioxidant pterostilbene pTeroPure
- OPKO distribution rights in Latin America

* As of March 31, 2012